Essential Testing in AML Care

**Molecular Testing (genetic or biomarker testing):** Laboratory testing that identifies certain gene mutations, proteins, chromosomal abnormalities and/or other molecular changes that are unique to an individual’s disease. In cancer, it may be used to evaluate treatment or to make a prognosis.

**Targeted Therapy:** A type of personalized medicine that works by blocking specific mutations and by preventing cancer cells from growing and dividing, without affecting normal cells.

### Genetic Testing Methods

**Karyotyping:** Testing of blood, bone marrow, or tissue in order to identify changes in chromosomes.

**Fluorescence In Situ Hybridization (FISH):** A chromosome test used to identify specific genes or chromosome changes.

**Next-Generation Sequencing (NGS):** Technology to sequence DNA or RNA to identify genetic variations associated with diseases or other biological phenomena.

### Common AML Mutations

<table>
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<tr>
<th>IDH (Isocitrate Dehydrogenase) Mutations:</th>
<th>NPM1 (Nucleophosmin-1) Mutation: The most common molecular mutation identified in adult AML.</th>
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<tr>
<td>Mutations in IDH1 or IDH2 are detected in approximately 20 percent of patients with AML.</td>
<td>TP53 (tumor protein 53) Mutation: A gene that helps stop the growth of tumors. TP53 is the most frequently mutated gene in human tumors. TP53 mutation rates are low in AML.</td>
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<td><strong>FLT3 Mutation:</strong> FLT3 stands for Fms-like tyrosine kinase. This gene mutation occurs in approximately 30 percent of AML patients.</td>
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### Approved FLT3 Inhibitors

- Gilteritinib (Xospata)
- Midostaurin (Rydapt)
- Sorafenib (Nexavar)

### Approved IDH Inhibitors

- Ivosidenib (Tibsovo)
- Olutasidenib (Rezlidhia)
- Enasidenib (Idhifa)

**Venetoclax (Venclexta):** Inhibitor therapy that targets the Bcl-2 protein. Venetoclax is being used in combination with hypomethylating agents (azacitidine [Vidaza] or decitabine [Dacogen]) to treat older AML patients who are not candidates for intensive induction therapy.

### Glossary Terms

**AGILE Study:** Global, Phase III, multicenter, double-blind, randomized, placebo-controlled clinical trial to evaluate the efficacy and safety of AG-120 (ivosidenib) + azacitidine vs placebo + azacitidine in participants with previously untreated acute myeloid leukemia (AML) with an IDH1 mutation.

**Bone Marrow Biopsy:** Procedure that involves collecting a small sample of bone marrow, usually from the hip bone, in order to be examined by a laboratory. This procedure is used to confirm a diagnosis and may be used to monitor the disease over time.

**Menin Inhibitors:** A novel class of targeted therapies showing promise in the treatment of acute leukemias with them NPM1 mutation or the KMT2A mutation.

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